

RPDD may be granted to drugs in development for diseases which primarily affect children under the age of 18, have an incidence of less than 200,000 new cases per annum in the US and which are serious or life-threatening.

Paxalisib trial

In October 2018, St Jude Children's Research Hospital in Memphis, Tennessee, began a phase I clinical trial of paxalisib in DIPG (NCT03696355).

This study reported favourable top-line safety data in September 2019 and established 27 mg/m² as the maximum tolerated dose for paediatric use.

The study has completed recruitment and initial efficacy data is anticipated during the second half of 2020.

This data will be used to guide future development of paxalisib (formerly GDC-0084) in this disease.

Extensive research

Dr Matt Dun and colleagues at the University of Newcastle, Australia have conducted extensive laboratory research with paxalisib, focused on phosphoproteomic analysis of its activity in DIPG cell lines.

Phosphoproteomics is a new approach in cancer research that attempts to discern how complex signalling pathways are modified in tumours.

Work at the Dun laboratory has shown paxalisib to be broadly active in DIPG and has identified a number of potential combination strategies which may enhance its activity.

Initial data was presented at the Society for Neuro-Oncology (SNO) Pediatric Neuro-Oncology Basic and Translational Research Conference in San Francisco, CA, in May 2019.

Further ongoing work in animal models is expected to provide additional insight.

In parallel, related laboratory research is underway in the DMG Research Center at the University of Zurich, Switzerland, under the leadership of Dr Javad Nazarian.

Dr Nazarian is also the principal investigator at Center for Genetic Medicine within the Children's National Medical Center, Washington DC with a focus on DIPG.

Laboratory research is also being conducted at St Jude Children's Research Hospital by Dr Chris Tinkle and Dr Suzanne Baker and colleagues, in parallel to the ongoing phase I clinical trial at that centre.

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